Off-Label Drug Use

I. DESCRIPTION
The FDA approves drugs for specific medical indications, however, these approved drugs are often found to be effective for indications not approved by the FDA. The pharmaceutical companies frequently do not seek FDA approval for these "off-label" indications.

II. CRITERIA/GUIDELINES
A. Precertification is required for the off-label use of an FDA-approved drug when HMSA has a medical policy for that drug. Click for Medical Policies.

B. Off-label use of an FDA-approved drug will be covered for the specific off-label indication when the drug is clearly stated to be effective for the specific off-label indication in one of the references listed below.
   1. Micromedex Drugdex evaluations when Strength of Recommendation is Class I or IIa
   2. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium and corresponding Clinical Practice Guidelines when Category of Evidence and Consensus is 1 or 2A (Note: for oncology drugs)

C. If the drug is not clearly stated to be effective for the off-label indication in one of the above references, the requesting provider must submit published scientific evidence and/or professional standards of care supporting the use of the drug for the requested off label indication.
   1. Scientific evidence means controlled clinical trials that demonstrate the effect of the intervention on health outcomes published in peer-reviewed literature.
   2. If controlled clinical trials are not available, other studies that demonstrate a causal relationship between the intervention and the health outcomes will be considered.

D. If the drug is not clearly stated to be effective for the off-label indication in one of the above references, the drug will be covered (subject to Limitations and Administrative Guidelines) when the drug:
   1. Is for the purpose of treating a medical condition;
   2. Is the most appropriate delivery or level of service, considering potential benefits and harms to the patient;
   3. Is known to be effective in improving health outcomes; provided that:
a. Effectiveness is determined first by scientific evidence;
b. If no scientific evidence exists, then by professional standards of care; and
c. If no professional standards of care exist or if they exist but are outdated or contradictory, then by expert opinion; and

4. Cost-effective for the medical condition being treated compared to alternative health interventions, including no intervention. For purposes of this paragraph, cost-effective shall not necessarily mean the lowest price.

III. LIMITATIONS
A. When approving an off-label use of an FDA approved drug, it is appropriate to consider coverage based on a trial of therapy concept. For example, a drug may be approved for 90 days during which time the patient’s measurable response to therapy will be assessed. Specific performance measures must be identified by the ordering physician and an HMSA Medical Director prior to commencement of a trial of therapy.
B. If HMSA has a policy regarding the medical necessity of a specific drug, the request should be reviewed using that policy first. If the requested indication is not addressed in the specific policy, then the request will be reviewed using the Off-Label Drug Use policy.
C. Coverage is not provided for experimental and Drug Efficacy Study Implementation (DESI) drugs for HMSA QUEST plan members.
   1. Drug Efficacy Study Implementation (DESI) drugs are those drugs first marketed between 1938 and 1962, which were approved as safe but did not require that effectiveness be shown prior to FDA approval. The DESI program subsequently made a determination of fully effective for most of these products and they remain in the marketplace. A few DESI products remain classified as less than fully effective while awaiting final administrative disposition. Also classified as DESI, are many products listed as identical, similar, or related to actual DESI products.

IV. ADMINISTRATIVE GUIDELINES
A. Precertification is required for the off-label use of drugs for which HMSA has a medical policy including the policy Specialty Drugs Requiring Precertification. Click for Medical Policies.
B. To precertify, please complete the fax form as indicated.
C. The following must be submitted:
   1. Clinical information supporting the diagnosis
   2. Proposed treatment plan
   3. Published scientific literature or professional standards of care supporting the use of the drug for the requested off-label indication
D. Precertification is required for extension of treatment beyond the initial approval period. Current clinical documentation (e.g., office visit notes and applicable studies, supporting a response to treatment) must be submitted.
V. IMPORTANT REMINDER

The purpose of this Medical Policy is to provide a guide to coverage. This Medical Policy is not intended to dictate to providers how to practice medicine. Nothing in this Medical Policy is intended to discourage or prohibit providing other medical advice or treatment deemed appropriate by the treating physician.

Benefit determinations are subject to applicable member contract language. To the extent there are any conflicts between these guidelines and the contract language, the contract language will control.

This Medical Policy has been developed through consideration of the medical necessity criteria under Hawaii’s Patients’ Bill of Rights and Responsibilities Act (Hawaii Revised Statutes §432E-1.4), generally accepted standards of medical practice and review of medical literature and government approval status. HMSA has determined that services not covered under this Medical Policy will not be medically necessary under Hawaii law in most cases. If a treating physician disagrees with HMSA’s determination as to medical necessity in a given case, the physician may request that CVS/caremark reconsider the application of the medical necessity criteria to the case at issue in light of any supporting documentation.

VI. REFERENCES


Revised: August 2016.